



Targeting Critical Regulators of Cancer Stem Cells

Grant Award Details

Targeting Critical Regulators of Cancer Stem Cells

Grant Type: Quest - Discovery Stage Research Projects

Grant Number: DISC2-12342

Project Objective: To identify a lead small molecule inhibitor of the RNA binding protein Musashi (MSI) to block the

growth and propagation of pancreatic or leukemia cancer stem cells

Investigator:

Name: Tannishtha Reya

Institution: University of California, San Diego

Type: PI

Disease Focus: Cancer, Pancreatic Cancer, Solid Tumors

Human Stem Cell Use: Cancer Stem Cell

Award Value: \$1,148,264

Status: Active

Grant Application Details

Application Title: Targeting Critical Regulators of Cancer Stem Cells

Public Abstract:

Research Objective

We will develop a small molecule inhibitor that blocks the growth of human pancreatic cancer and AML cancer stem cells in vitro and in vivo.

Impact

This work will lead to a new treatment for cancer stem cell driven diseases such as AML and pancreatic cancer. In addition, it will improve the prognosis and stratification of patients.

Major Proposed Activities

- Assay Validation: 1) Transfer of activity assays to CRO; 2) Development of binding assay at
- Hit validation: 1) Confirm top hits from primary screen in activity assays; 3) Profile top hits in binding assay; 4) Test top hits in cell-based functional assays
- Hit series prioritization: 1) Synthesize modified hit compounds; 2) Profile compounds in activity assays; 3) Profile compounds in binding assay; 4) Profile potent compounds in ADME assays
- Conditional knockout (KO) mouse generation and characterization
- Lead series nomination: 1) Test active compounds/inactive controls in cell-based functional assays; 2) Confirm MoA in cell-based assays; 3) Test top compounds in PK assay in mice (IV and oral)

California:

Statement of Benefit to Because this research will lead to development of new treatments for leukemia and pancreatic cancer, the State of California and its citizens will directly benefit. Pancreatic cancer affects people of all genders, ethnicities and socio-economic status. And while AML is the most common adult leukemia, it also accounts for more than 50% of all leukemia-associated mortality in children. Thus, if successful, the new therapeutic will improve outcomes for patients throughout the State of California.

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